



# Channeling In

The Newsletter of the IDEA League

## Critical Drug Stiripentol Receives Orphan Drug Status in the US

By Joan Skluzacek and Karen Glenn

The IDEA League is delighted to share the news that the US Food and Drug Administration (FDA) has officially designated stiripentol (Diacomit™) as an orphan drug for the treatment of Dravet syndrome, also known as Severe Myoclonic Epilepsy of Infancy. Stiripentol is the only medication currently indicated for use in Dravet syndrome and was approved as such by the European Medical Association in 2006. It has proven to be effective for improving seizure control in many children when used in combination with certain other anticonvulsants, most notably valproate and clobazam. Stiripentol works, at least in part, by increasing the efficacy of these other medications.

To date, stiripentol had not been approved by the FDA or any governmental regulating body outside of Europe. For this reason, it has rarely been covered by private or government-provided medical insurance. For most IDEA League families living outside of the European Union, the cost of stiripentol had skyrocketed, becoming a significant out-of-pocket expense. In some cases, families have been forced to reduce or eliminate their child's dose of stiripentol, resulting in increased seizures and life-threatening status epilepticus.

In response to this crisis, IDEA League leaders, past and current, including Joan Skluzacek, Michelle Welborn, and Mary Anne Meskis have

been working with Biocodex, the maker of stiripentol, to increase the availability and affordability of this drug. Biocodex agreed to apply for Orphan Drug Status within the US and arranged for KM Pharmaceutical Consulting, LLC, of Washington D.C., to represent them in this process. In May of this year, Joan, Mary Anne and Michelle met with Kathleen Clarence-Smith, MD, PhD, Co-founder of KM Pharmaceutical, along with her colleague, Benjamin P. Lewis, PhD, RPh, RAC, of Lewis Regulatory Consulting, LLC, who then began developing the application to the FDA. Conscious of the importance of this effort for children and families struggling with Dravet syndrome, they worked efficiently, collaborating with the IDEA League to gather data to support their case. On Monday, November 10, 2008 they contacted our Board of Directors with the exciting news.

Achieving orphan status increases the likelihood that stiripentol will be covered by public and private health insurance for treatment of Dravet syndrome. While under orphan drug status, the procedure for acquiring stiripentol will not change, but families are encouraged to list stiripentol as an orphan drug when applying for possible coverage through their insurance program. Orphan drug status is a major step toward the goal of eventual full FDA approval. Biocodex has agreed to pursue this end,

and the process for full approval has already been initiated. The help of IDEA League members within the US may be an important part of this process, as it is possible that one or more clinical trials may be a necessary.

"I feel so grateful for the hard work and dedication put forth by our Board of Directors, KM Pharmaceuticals, and by the members of the IDEA League. This is a monumental achievement that is going to make it much easier to improve the lives of children with Dravet syndrome all over the United States. This accomplishment is just an example of what is coming in the very near future. The IDEA League is now poised to make a substantial impact on the diagnosis, treatment and outcome of this devastating syndrome." says Angela Black, MD, Chair of the IDEA League Professional Advisory Board

Sincere appreciation goes out to Drs. Clarence-Smith and Lewis and to Biocodex for their willingness to collaborate on this important achievement, as well as to the members of the IDEA League who contributed their stories. It is our hope that this medicine will become increasingly accessible to the many children for whom it is essential.

## Special Edition!

November 11, 2008

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**IDEA** League  
International Dravet Syndrome Epilepsy Action

Our IDEAL is a Cure

## IDEA League Attends CNS Conference

By Karen Glenn

On November 5-18, 2008, the Child Neurology Society held its annual conference in Santa Clara, California, USA. The Child Neurology Society is a non-profit professional association of pediatric neurologists in the United States, Canada, and worldwide devoted to fostering the discipline of child neurology and promoting the optimal care and welfare of children with neurological and neurodevelopmental disorders.

The IDEA League sent several representatives to this conference, including Founder

Joan Skluzacek, President Laura Cossolotto, and Professional Advisory Board Chair Angela Black, MD. These representatives assisted at the IDEA League's booth, meeting and greeting numerous pediatric neurologists and other interested professionals, informing them about the IDEA League and its goals and objectives. They also attended portions of the conference, including a dinner symposium—Advances in the Identification and Management of Refractory Childhood Epilepsy Syndromes: Focus on SCN1A,

at which Dr. Jim Wheless of the University of Tennessee Health Science Center and Le Bonheur Children's Medical Center, included a slide and information about the IDEA League in his presentation.

The IDEA League is dedicated to increasing the awareness and appropriate treatment of Dravet syndrome and related sodium channel epilepsies. Many thanks to IDEA League Secretary Lori O'Driscoll for coordinating our organization's participation in this event, and to those volunteers who attended the CNS conference.



*The IDEA League is dedicated to increasing the awareness and appropriate treatment of Dravet syndrome and related sodium channel epilepsies.*

## Letters From Santa

The Holidays, with all their sparkle, are almost upon us! Wouldn't they be even a little brighter with a personalized letter from Santa himself? Now you can have a special note sent directly to your child with Dravet syndrome—or any other 'believer' in your life. All proceeds from this fundraiser will go directly to the IDEA League. Please contact Kim Skriba at [kim.s@idea-league.org](mailto:kim.s@idea-league.org) (US) or Marie Baker at [marie.b@idea-league.org](mailto:marie.b@idea-league.org) (international) for more details.



## Blocks of Love

By Kim Skriba

IDEA League member Brenda Young Ferrell, PhD, mother to Brian T. Ferrell (15) with Dravet syndrome has, through the years of staying home with her son, developed the fine hobby of quilting. Brenda is very excited to help the IDEA League spread awareness of Dravet syndrome and has generously offered to make a gift that will represent our Dravet children in a very special way: a Dravet Quilt.

The Dravet Quilt will be a collection of 8 inch square 'blocks', each created by people who have been affected by this rare form of epilepsy. Designs for the

blocks can be embroidered, painted, screen printed, and more. Says Brenda, this quilt will be a "CELEBRATION of our Dravet kids."

After all the blocks have been received, Brenda will assemble them into one large quilt that will bring recognition to Dravet syndrome, as well as to those who suffer from this challenging condition. The quilt will be displayed at national and international events, fundraisers, and any where else that the IDEA League Board deems appropriate. As the quilt travels, it will be accompanied by a notebook of

'Companion Letters', as well as graphs and maps plotting where the quilt blocks have come from, where they have been displayed, and more.

Details of this project are still being finalized. If you are interested in contributing to the quilt, the first step is to email Kimberly Skriba at [skriba\\_k@bellsouth.net](mailto:skriba_k@bellsouth.net). The quilt blocks will need to be completed and returned by January 1, 2009 so that the quilt can be assembled and ready for display at the National Walk for Epilepsy in Washington, DC, USA in March of 2009.

*...this quilt will be a "CELEBRATION of our Dravet kids."*  
—Brenda Young Ferrell

